Picking Biotech Stocks in the "COVID-19" Era

JOHN MCCAMANT

EDITOR, ***MEDICAL TECHNOLOGY STOCK LETTER***

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MTSL & BioInvest

- The ***Medical Technology Stock Letter*** is your best tool for understanding and investing in Biotech stocks
- Get educated, stay informed and profit from Biotechnology
- BioInvest is our one-stop-shop website that answers all of your biotech industry questions
- Visit bioinvest.com

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Today's Presentation

- Overview Of MTSL
- What MTSL Brings To The Table
- Biotech Metrics
- Picking Biotech Stocks in the "COVID-19" Era
- Platform Technologies
- ***Why you need to subscribe to MTSL***

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MTSL Model & Trader's Portfolios

- Long-term horizons
- Mix of Tiers (1,2,3) based on market cap/relative risk/stage of development
- Seven-Year CAGR Performance, 32% in Model & 62% in Trader's Portfolios
- In 2013, the MTSL Trader's Portfolio delivered the #1 Portfolio Performance of >500 portfolios followed by Hulbert's Financial Digest
- 2016, only year out of last seven with a negative return despite significant sector volatility

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BENCHMARKS

	NASDAQ	S&P 500	MODEL	TRADER'S
Last 2 Weeks	4.1%	4.0%	5.9%	16.2%
2020 YTD	-0.9%	-9.9%	-8.3%	-0.6%
Calendar Year 2019	35.2%	28.8%	10.7%	44.1%
Calendar Year 2018	5.7%	6.6%	4.5%	11.2%
Calendar Year 2017	29.3%	19.9%	65.6%	98.9%
Calendar Year 2016	7.5%	9.5%	-29.6%	-30.5%
Calendar Year 2015	-0.1%	-0.1%	25.1%	27.9%
Calendar Year 2014	13.4%	11.4%	29.2%	45.0%
Calendar Year 2013	38.3%	29.6%	103.4%	214.7%

MTSL Portfolios - Performance 2012-2019 YTD

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Who is MTSL?

- MTSL is John McCamant & Jay Silverman
- Dynamic Biotech analyst team
- Over 60 years analytical experience
- Objective analysis (no banking fees, etc.)
- Early recommendations became leading companies
  - INCY, BMRN, PCYC, CELG, DNA, MDCO
- Current ones can too…..
  - ESPR, MDGL, NKTR, SGMO, PGEN, etc.

#1 and #1 Team Up

John McCamant

Jay Silverman

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John McCamant

- Joined the *MTSL* as Associate Editor in 1987, named Editor of this leading investment newsletter in August 2000
- 30 years on the front lines of biotechnology investing. As an equities analyst for the American Healthcare Fund
- At Burrill & Company, a San Francisco-based private merchant bank, he was a lead in raising $75 million for a venture capital fund
- Consulted and quoted by The Washington Post, Business Week, Reuters, Bloomberg, CBS MarketWatch

Jay Silverman

- Senior biotechnology and pharmaceutical analyst for >30 years and has a tremendous track record in analyzing and recommending biotech stocks
- Ranked #1 in Biotechnology by The Wall Street Journal's Best on the Street annual analyst survey
- Frequently quoted in The Wall Street Journal, TIME, Fortune and featured on CNBC and CNN
- VP Business Development for Thorne Research from 2010 – 2012, a leading natural products company (thorne.com)
- Joined MTSL in 2013

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BIOINVEST.com

- Primary Feature: **BSA, The White Papers**
- Proprietary **CEO** Insights
- MTSL ***archives available*** online for subscribers only (hundreds of past Issues)
- Comprehensive industry event **calendar**
- Investment philosophies laid out **Issue by Issue**

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MTSL - The BSA
A Snapshot of The Present/Future

- Each Bi-Weekly Issue of MTSL begins with the Biotech Sector Analysis (BSA)
- Macro picture of the near-term state of the industry
- **Assesses investor sentiment**
- **Quite useful for understanding the near-term fundamentals moving the biotech stock market**
- **Used by traders/technicians for short-term trades/sector moves**

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MTSL - The BSA
A Snapshot of The Present/Future

- Major industry events (whether MTSL stocks or not)
  - That have occurred since the previous Issue
  - That will or expect to occur over the next ~2-4 weeks
- **Regulatory - Key FDA approvals, PDUFA dates, data releases**
- **M&A/deals - major driver of stock prices**
  - **Which themes are hot? e.g., gene therapy, RNAI, COVID vaccines, immunotherapy/oncology**
- **Technicals - charts often tell the story**
  - **Support/resistance levels**

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MTSL - CLINICAL TRIALS WATCH

- Every Issue, MTSL publishes new clinical trials and clinical trial updates of and/or related to our recommended stocks
- Direct links to the posts on clinicaltrials.gov
- Keeps our subscribers well-informed and ahead of the game

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MTSL - CLINICAL TRIALS WATCH

Clinical Trials Watch

Relevant New Studies or Changes Posted on ClinicalTrials.gov for our MTSL Portfolio and/or Related Companies S

COVID-19 TRIALS

ABBV – Treatments for COVID-19: Canadian Arm of the SOLIDARITY Trial (CATCO)

REGN – Sarilumab COVID-19

REGN – Evaluation of the Efficacy and Safety of Sarilumab in Hospitalized Patients With COVID-19

Population Health Research Institute – Anti-Coronavirus Therapies to Prevent Progression of Coronavirus Diseas

University of OXFORD – A Study of a Candidate COVID-19 Vaccine (COV001)

GILD – Expanded Access Treatment Protocol: Remdesivir (RDV; GS-5734) for the Treatment of SARS-CoV2 (CoV) Inf

INCY/LLY/Hospital of Prato – Baricitinib in Symptomatic Patients Infected by COVID-19: an Open-label, Pilot Study.

INCY – Study of the Efficacy and Safety of Ruxolitinib to Treat COVID-19 Pneumonia

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BIIB/IONS – Observational, Postmarketing Surveillance Study of Spinraza Injection (Nusinersen Sodium)

CELG/BMY – A Study of CC-90010 in Combination With Temozolomide With or Without Radiation Therapy in Subje Glioblastoma

KITE/GILD/Humanigen – Study of Lenzilumab and Axicabtagene Ciloleucel in Participants With Relapsed or Refrac 19)

VSTM – Duvelisib Maintenance After Autologous Stem Cell Transplant in T-Cell and Indolent B-Cell Lymphomas

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MTSL - COMPANY UPDATES

- Every Issue, MTSL publishes new analyses of companies in our Portfolio Universe
- Clinical, regulatory, partnership, competition and/or financial announcements
- Analyze the present and predict the future
- Looking ahead as to what to expect big/small pictures
- 24 issues per year!
- Easy to read, for the layman as well as the scientist, physician, industry executive

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MTSL - WHITEPAPERS

- Several times a year, we will publish in-depth whitepapers on the latest relevant themes of biotech investing
- For example, hemophilia, gene therapy, etc.
- Timely - forward looking
- Often accompany key MTSL recommendations
- Again easy to read for the layman as well as the scientist, physician, industry executive

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MTSL - BREAKING NEWS

- We often send subscribers “BIOINVEST NEWS” announcements between Issues when key events occur
- For example, we recently sent an email like the one below:
  - **BioInvest News - Sangamo - SGMO** - BIIB Steps Up Big With Global Neuro Gene Editing Deal - BUY - After the close, Sangamo announced a worldwide deal with Biogen for gene regulation therapies in neurology……
  - Even more timely - value added content
- Keeping subscribers well-informed and up-to-date especially on our covered companies

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Biotech Blockbusters in 2019

- Humira-$16.1 Billion
- Harvoni-$9.1 Billion
- Remicade-$7.8 Billion
- Rituxan-$7.3 Billion
- New Ones - MTSL Recommendations
  - Jakafi - $2.1 billion
  - Spinraza - $2.2 billion

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Key Drug Pricing Proposals

- Senate Finance bill – full text expected over the near term, potential re-introduction of Medicare Part D rebate rule
- House Speaker Pelosi's bill – Released on 9/19, key items included direct Medicare negotiations for the 25-250 most costly drugs in the US, with prices available to both Part B/D and private payors, borrowing from the IPI to set negotiated prices, Part D out-of-pocket spending cap, and inflation caps
- Lots still up in the air - changing of the guards and COVID delays/reimbursements
- Joe Biden endorses Obamacare (now that Warren/Sanders “Medicare For All” are out)
- Bi-partisan attacks likely come election time

Does Innovation Trump High Drug Prices?

- Starting in 9/15, Clinton Tweets About Drug Pricing Signaled Top of Biotech Historic Rally
- Until COVID - biotech stocks lagged the overall markets
- High Drug Prices Blamed Throughout Primaries By Everyone
- Trump Tweets Had Also Been Hard on Drug Pricing/Biotechs

Trump - Maybe Good For Biotech?

- Generally, markets love Trump as Pro Business
- Tax repatriation had significant positive for drug/bios cash hoards
- Regulatory so far, so good – Hahn off to strong start
  - From MD Anderson
  - well-respected immuno-oncologist
- FDA is critical for biotech
  - Ultimate gatekeeper to huge marketplace of semi-monopolies
- However, details matter
  - e.g., biosimilars, gene therapy guidelines

COVID-19 PRIMER

* VIRUS TESTS-still lacking, hundreds of millions will be needed annually
* ANTIBODY TESTS-Enable to test for previous infection, asymptomatic status, vaccine protection/antibody titers
* TREATMENTS-GILD's Remidisvir first part of solution to puzzle, similar to AZT for HIV
* VACCINES-Holy Grail to treat viruses, hardest of four to develop by far

COVID-19 PRIMER

* VIRUS TESTS-still lacking, hundreds of millions will be needed annually. Very important tool for information/tracking COIVID-19.
* When combined with Contact Tracing allows for much better decision making

COVID-19 PRIMER

* ANTIBODY TESTS-Enable to test for previous infection, asymptomatic status, vaccine protection/antibody titers. Also allows for testing of antigen drift as the virus mutates. Asymptomatic disease status will be very important with current estimates of 20-60% harboring the virus without showing signs of being sick. Potential “green cards” if antibody tests can prove accurate. Current tests use blood, saliva may easier and COVID-19 may be more prevalent in mucosa than blood.

COVID-19 PRIMER

- TREATMENTS-GILD's Remidisvir first part of solution to puzzle, similar to AZT for HIV where a cocktail of usually three different medications to keep virus at bay. We will need more options. Rapid emergency use approval - limited to hospitals/severe patients
- INCY's Jakafi has potential to treat "cytokine storm" in COVID-19 patients. Already approved pill with very good safety profile. Pills more flexible than IV treatments like Remidisvir
- REGN therapeutic/prophylactic antibodies
- Already approved drugs, Jakafi, and remidisvir that were stopped in clinical development for lack of efficacy, can be fast tracked into COVID-19 trials as they have already been proven safe in both animals and humans - multiple trials underway
- Hydrochloroquine also in many clinical trials - mixed results so far
- Need to be careful of COVID-19 press release effect as literally 100's of companies making claims in treatment and vaccine development

COVID-19 PRIMER

* VACCINES-Holy Grail to treat viruses, hardest of four to develop by far
* Fastest time line for vaccines four years for mumps
* HIV vaccine has eluded Fauci and the scientific community for 30 years and counting.
* The key to vaccines is having the right conserved target which over time demonstrates minimal mutation

COVID-19 PRIMER

* VACCINES
  * MRNA - RNA vaccine just cleared for Phase II study with NIH
  * Univ of Oxford/AZN - large Phase I underway
  * PFE/BNTX - just initiated first patients in Phase I
  * JNJ/BARDA
  * SNY/GSK
  * INO
  * NVAX - initial trials underway

COVID-19 PRIMER

- MTSL Companies Have Been Vetted For COVID-19 Impact in these main areas:
  - Potential impact on revenue/supply chain/inventory
  - Potential impact on clinical trials
  - Positive impact on buy-ins for Q1 earnings
  - Potential impact on 2020 Catalysts

COVID-19 PRIMER

* MTSL Companies Have Been Vetted For COVID-19 Impact In These Main Areas:
* Potential impact on revenue/supply chain/inventory to date has been manageable - Orphan Drugs (BMRN, IONS), gene therapy (SGMO)
* Revenue will be impacted but can bounce back fast - e.g., PCRX
* Biotech's focus on oncology and orphan disease has helped as more severe disease patients less likely to miss treatment.
* Cancer and MS are great examples of disease with minimal impact despite many treatments needing to be infused in a hospital setting

COVID-19 PRIMER

- MTSL Companies Have Been Vetted For COVID-19 Impact In These Main Areas:
- Most ongoing trials have seen minimal change as oncology and orphan diseases represent highly motivated patients who are used to much worse life threats than COVID-19
- Most new trials will be delayed until COVID-19 receeds
- Telemedicine has advanced significantly as industry adapts
- FDA becoming key as they have shown ability to adapt on the fly and demonstrating remarkable flexibility for a government agency

COVID-19 PRIMER

- MTSL Companies Have Been Vetted For COVID-19 Impact In These Main Areas:
- Potential impact on 2020 Catalysts remains to be seen.
- However, we have seen the FDA continue to grant approvals or even ahead of time.
- Deals are being struck with ESPR's Japanese deal with Otsuka
- Scientific conferences are being held virtually with full data presentations completely accessible for both scientists and Wall Street.
- ASCO and other key meeting (ADA, EHA) will be very interesting despite being held remotely this year

Current Reasons For M&A

- Drugs on the market
- Drugs in later development to augment pipelines
- Patent expiration which leads to biosimilar competition, particularly in Europe where the “writing is on the walls” for the future in US
- R&D Innovation is key
- COVID-19 Impact on cash, pre-commercial biotechs more likely to get bought with less access to capital markets
- Cash rich global players ready to take advantage with bolt-on deals

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M&A Deals

- 2 major biotech deals last year (after GILD/KITE, CELG/JUNO in 2017/18)
  - BMY-CELG ($85 billion)
  - LLY-LOXO ($11 billion)
- Nearly all the large drug companies are publicly talking about doing more deals, and we have already seen signs of Bidding Wars: AMGN, BIIB, CELG, GILD, NVS, MRK, PFE
- GILD & BIIB most likely to pull trigger as they both have recently suffered major blows to their respective pipelines
- Cash hoards keep building and external acquisitions have historically been the major driver of Big Pharma/Big Biotech growth with AMGN making 30+ acquisitions over the years
- so stayed tuned…..

- *……Despite COIVD lockdowns, two small deals were just announced at >100% premiums (ALXN/PTLA) and Menarini/STEM*

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M&A High for Access to Innovation

- Gene Therapy
- Gene Editing
- Cell Therapy
- Immune Oncology
- Antisense/RNAi

The pace of innovation continues to accelerate, and first-in-class and/or disruptive technologies continue to attract significant investor attention and capital

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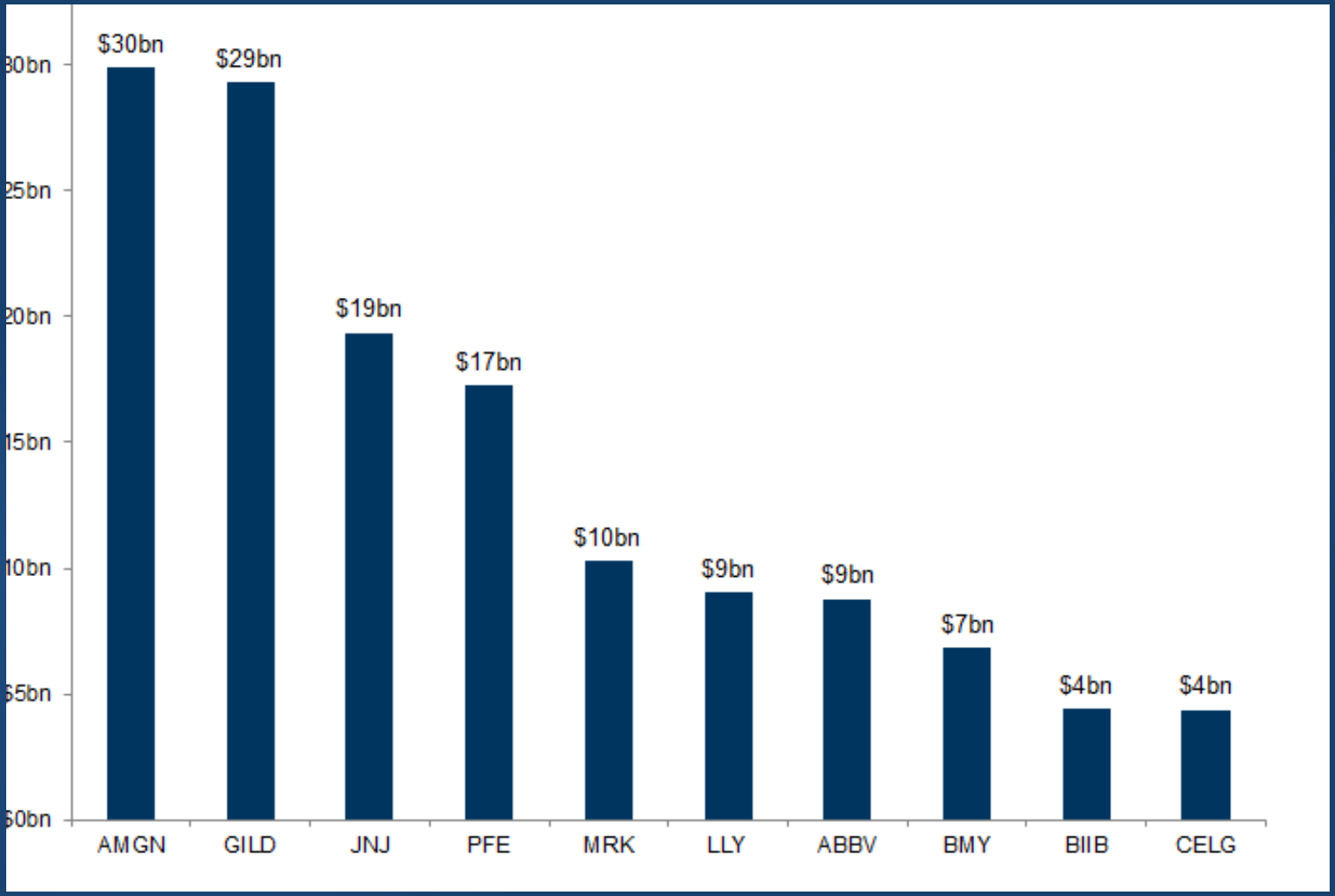
BLUE CHIP CASH HOARDS KEEP GROWING 2019*

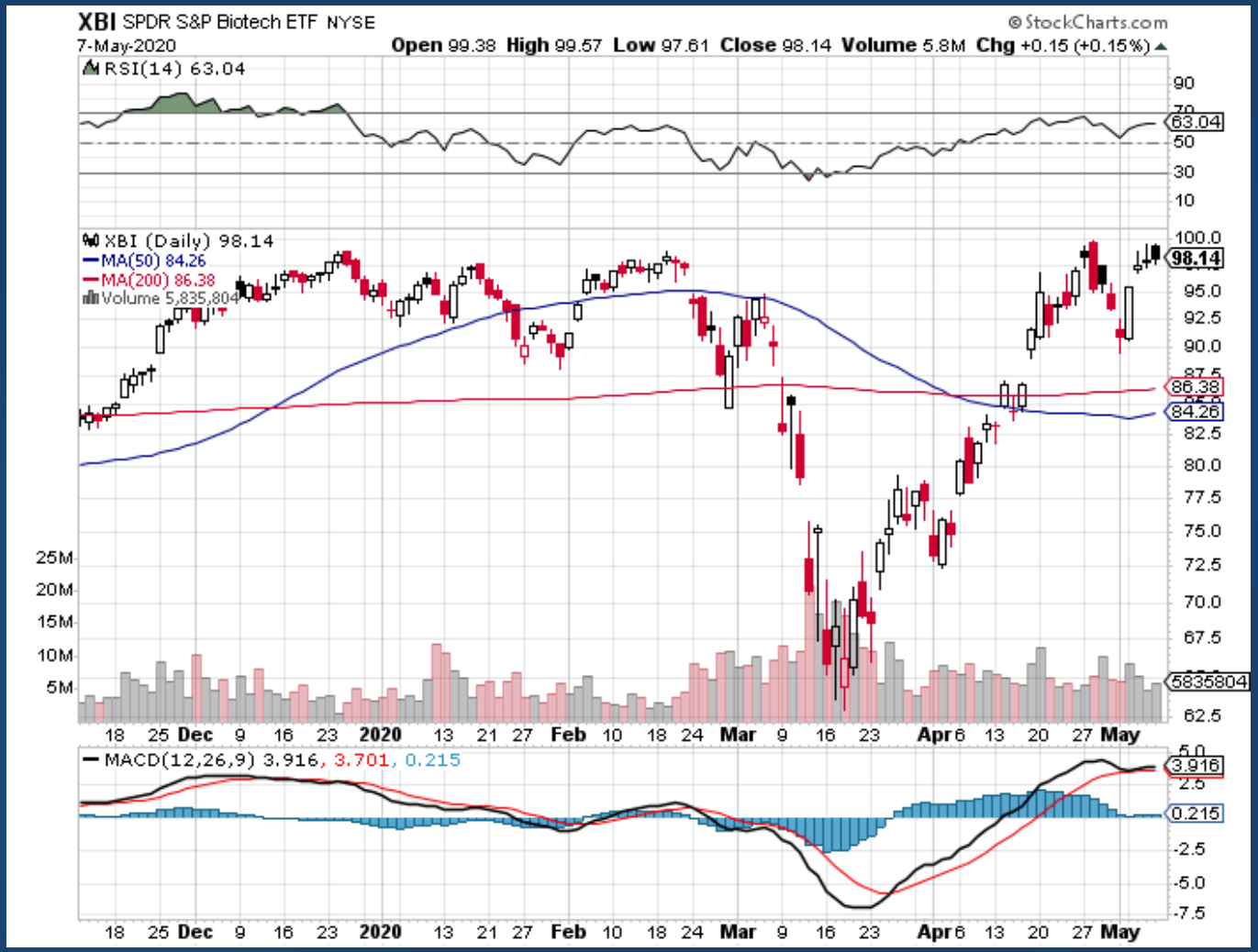


* Pre-mergers ABBV/AGN, BMY/CELG

BULL CASE FOR Biotech in 2020

- Biotech is part of the solution to both COVID-19 and future pandemic threats with a huge need for diagnostics, drugs and vaccines
- Investors have shunned biotech for two-plus years before COVID-19 on drug pricing fears/election rhetoric.
- The pandemic counters this negativity by shining a positive light on the industry and its tremendous potential to be part of the solution.
- “Good drugs will be used and paid for” (e.g., quote from leading neurologist)
- Profitable Big Biotech stocks have gone from market P/E discounts to market multiples since February
  - This had been the case (i.e., premium multiples) from ~1980-2010

BULL CASE FOR Biotech in 2020



XBI Catches Up To NASDAQ

Biotech Recovery Continues

- M&A remains an important trigger for SMID-cap biotech. Numerous big companies have said they are looking at lots of targets, bolt-on acquisitions
- COVID-19 will make it harder for some companies to finance
- Big Bio and Pharma continue on a spending spree since mid-2019
- e.g., BIIB and GILD and ALXN starving for new drugs after clinical setbacks and slowdowns in key blockbusters
- Global demographics/demand rising
- Pricing environment still OK despite increasing scrutiny (not many serious issues from Cos; e.g., holding 9.9% price increases)

Key drivers that support our positive long-term fundamental view

- Secular industry fundamentals are still solid: we know more about diseases, targets, and designing better targeted drugs than ever before, COVID-19 vividly illustrates this
- Innovative industry pipelines are clearly emerging and exciting (immuno-oncology/targeted cancer therapy, gene therapy, Alzheimer's, neurology, NASH, Orphan drugs/rare diseases, etc.)
- Worldwide prescription drug sales are expected to be >$1 trillion by 2020 with growing contribution from biotech drugs

Key drivers that support our positive long-term fundamental view

- Cell, gene and nucleotide therapies more than doubled over the past three years - though they still represent less than 10% of the late-stage pipeline
- Emerging biopharma companies (those that spend less than $200 million annually on R&D and less than $500 million in sales) account for 72% of all late-stage pipeline activity, up from 61% a decade ago
- Big Pharma still spends a huge amount on R&D: The top 15 largest companies spent more than $100 billion for the first time last year, but the data show large pharma companies (those with more than $10 billion in yearly drug sales) have seen their R&D share drop from 31% to just 20% in the past decade
- Independent public bios have competitive cash positions
- For example, IONS ($2.4 billion) and NKTR ($1.5 billion) and others

Weekly NBI Performance

Various Biotech Innovation Technology Platforms

- Gene Therapy:
  - ABEO, AGTC, AVXS, BLUE, **BMRN,** BOLD, DMTX, ONCE, RGNX, **SGMO,** VYGR, QURE
- CAR-T:
  - BLUE, GILD/KITE, BMY/CELG/JUNO, NVS, **PGEN, ZIOP**
- T-Cell and ImmunoOncology (I/O):
  - ADAP, ADRO, ATRA, NK, ABBV, ADXS, DVAX, **FPRX, INCY,** MGNX, **NKTR,** NLNK, SGEN, TSRO, **PGEN**, **ZIOP**
- RNAi/Antisense:
  - ABUS, ALNY, ARWR, **IONS**, MRNA, RGLS, WaVe
- Gene Editing:
  - NTLA, EDIT, CRSP, PRQR, **SGMO**

PLENTIFUL PLATFORMS

- What Makes a Good Platform Technology?
  - Breadth & Depth
  - Management Execution
  - Intellectual Property

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Benefits of Platform Technologies

- Sound and Multiple Partnerships
- Lower Risk through Diversification
- Financial, Infrastructure Benefits (e.g., clinical, regulatory, marketing, etc.)
- More Opportunities for Management to Execute
- License out initial compounds, retain full rights for independence, exponential growth over time

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MTSL Platform Companies

- **ALKS** – Long Acting Anti-Psychotics
- **FPRX** – Next Generation Mabs & Proteins
- **INCY** – Small Molecule Chemistry
- **IONS** – Antisense
- **NKTR – Sustained Release Polymers**
- **SGMO- Gene Editing, Gene & Cell Therapy**
- **ZIOP/PGEN – Cell Therapy for Cancer**

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Behind Every Pipeline

- Lock Solid Intellectual Property
- Validating Partnerships
- Quality Management
- Solid Cash position

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Pipelines = Catalysts

- Catalysts are the lifeblood of Biotech and represent value creation opportunities; a deep pipeline allows for more catalysts
- Data is key and positive clinical data adds value as a drug advances in development
- Partnerships are vital for validation, infrastructure support, and risk diversification

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2000 versus 2020: What has changed?

- In 2000 Platform companies sold their tools leaving little future growth
- In 2020 Platform companies use their tools to find and develop new drugs to become pipeline companies
- Today's management teams much better, more experienced than in 2000
- New FDA commissioner has reduced approval times lowering R&D costs

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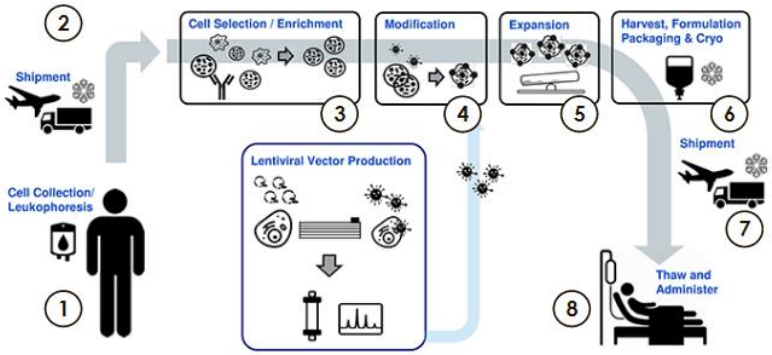
PRECIGEN

Our UltraCAR-T® Platform Promises a More Effective Way to Treat Patients

Conventional CAR-T

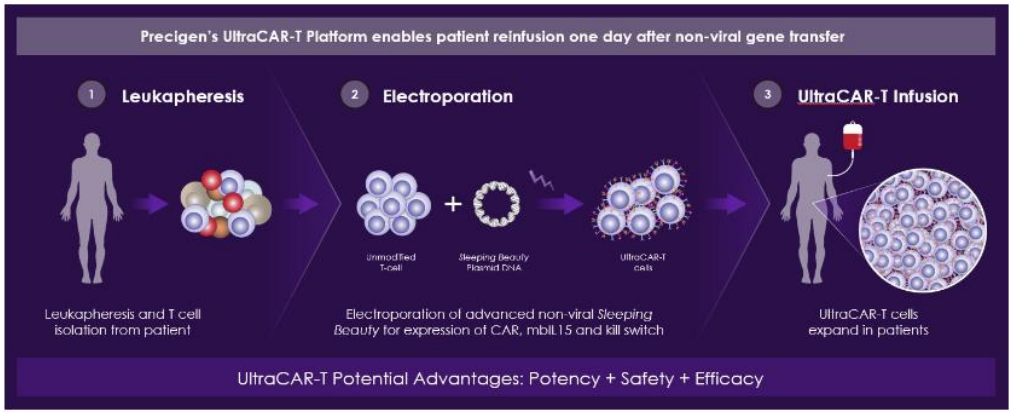
Viral vectors and *ex vivo* expansion result in long delays for patient treatment and high cost



- Reliance on viral vectors
  - Complexity of manufacturing viral vectors
- Long and complex CAR-T cell manufacturing process
  - Long delays for patients
  - High cost of manufacturing
- Exhausted T cell phenotype
- Major challenges in solid tumor treatment

UltraCAR-T

Overnight non-viral gene transfer eliminates long delays for patient treatment and lower manufacturing cost



- Non-viral gene delivery
  - Simplified manufacturing of Plasmid DNA
- Overnight UltraCAR-T manufacturing process
  - No *ex vivo* expansion necessary
  - Reduced manufacturing cost
- Stem-like memory T cell phenotype
- Enhanced potential for expansion and persistence

PRECIGEN

Robust Pipeline with Many Milestones to Drive Value

PRODUCT	PLATFORM	INDICATION	DISCOVERY	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	MILESTONES
AG019	ActoBiotics	Type 1 Diabetes						Interim data 3Q20
PRGN-3005	UltraCAR-T	Ovarian Cancer						Initial data 2H20
PRGN-3006	UltraCAR-T	AML, MDS						Initial data in 2H20
INXN-4001	Non-viral UltraVector	Heart Failure						Top line data 2H20
PRGN-2009	OTS AdenoVerse Immunotherapy	HPV* Solid Tumors						Initiate Phase 1 2020

ESPERION® The Lipid Management Company

~97 Million People in the United States Have High Levels of Bad Cholesterol

Did you know?

- **8.7 Million Patients in the U.S. Are Taking Statins, but Need Additional Therapy to Reach Their LDL-C Goals**
- **9.6 Million Patients in the U.S. with High LDL-C Are Not on Statins, often due to Tolerability Concerns**

Source: ZS Associates primary and secondary research, Sep-Oct 2018. Primary research N = 350 healthcare practitioners

ESPERION® The Lipid Management Company

Our First-In-Class Medicines now FDA-Approved

NEXLETOL™
(bempedoic acid) Tablets
are the first oral,
once-daily, non-statin LDL-C lowering medicine approved since 2002 for indicated patients

Now Available in U.S.

NEXLIZET™
(bempedoic acid and ezetimibe) Tablets
are the first oral non-statin, LDL-C lowering combination medicine ever approved

Available by July 2020 in U.S.

NEXLETOL™ and NEXLIZET™ are each indicated as an adjunct to diet and maximally tolerated statin therapy for the treatment of adults with heterozygous familial hypercholesterolemia (HeFH) or established atherosclerotic cardiovascular disease (ASCVD) who require additional lowering of LDL-C.

Limitations of Use: The effect of NEXLETOL™and NEXLIZET™ on cardiovascular morbidity and mortality has not been determined.

NEXLETOL™ and NEXLIZET™ available by prescription only.
Known as NILEMDO ™ (bempedoic acid) &
NUSTENDI ™ (bempedoic acid and ezetimibe) in Europe

Full prescribing information can be found on slides 24/25 and online:
https://pi.esperion.com/nexletol/nexletol-pi.pdf and https://pi.esperion.com/nexlizet/nexlizet-pi.pdf

ESPERION The Lipid Management Company

Partnering for Global Commercial Success

Companies with Proven Cardiovascular Excellence

Daiichi Sankyo Europe

(Product branded as NILEMDO™ & NUSTENDI™)

Daiichi-Sankyo

European Collaboration Overview:

- Largest EU agreement in history
- $900M in milestones plus royalties
- Marketing Authorization Approval Transfer to DSE in progress (Q2)
- Set for Q3 Launch for both medicines
- 1,000+ Cardiology-focused commercial team

Otsuka Pharmaceutical Co.

Otsuka

Japan Collaboration Overview:

- Largest Japan agreement in history
- +$600M in milestones and development costs plus royalties
- Otsuka responsible for development, regulatory approvals, and commercialization in Japan

Increasing productivity and realizing value through pharmaceutical partnerships

	Biogen	GILEAD	Pfizer	Pfizer	SANOFI	Takeda
Target/ therapeutic area	Neurological including AD, PD	Oncology anti-CD19 CAR-T	C9ORF72 ALS	Hemophilia A	Beta thalassemia, Sickle Cell disease	Huntington's disease
Development phase	Preclinical	Preclinical	Preclinical	Phase 3	Phase 1/2	Preclinical
Technology	Genome regulation	Cell therapy	Genome regulation	Gene therapy	Cell therapy	Genome regulation
Royalties (% on net sales)	High-single to sub-teen double-digit	Single-digit	Mid- to high-single digit	Low teens to 20	Double-digit	Single-digit
Upfront & equity	$125M payment + $225M in equity purchase	$150M payment + $50M in equity purchase	$12M	$70M	$20M	$13M
Milestones	Up to $2.37B ($925M pre-commercial, and $1.445B for 1st sale and sales thresholds)	Up to $3.1B ($1.3B through 1st sale, and $1.8B sales thresholds)	Up to $150M preclinical and commercial	Up to $475M ($300M for SB-525 and $175M other)	Up to $276M for both programs	-

Cash through license fees, milestones, and equity: **~$700 million***
Future opportunity: **Royalties** on net product sales, as well as **$6.34 billion** in potential milestone payments

* Including Biogen collaboration

Eliminated FVIII replacement use in high dose cohort

Factor VIII Replacement Usage

Dose Cohort (dose vg/kg)	Subject	Follow-up (weeks)	Factor VIII Prophylactic Regimen Prior to Dosing	Factor VIII Infusions ≥3 Weeks Following SB-525 Treatment
9e11	1	112	2/week	115
9e11	2	103	2/week	26
2e12	3	93	2/week	13
2e12	4	86	3/week	9
1e13	5	70	Every other day	17
1e13	6	61	Every other day	0
3e13	7	44	Every 4 days	0
3e13	8	37	Every other day	1*
3e13	9	24	Every 3 days	0
3e13	10	22	Every 3 days	0
3e13	11	12	2/week	0

*Prophylactic coverage stopped 3 weeks and 2 days after SB-525 administration.
Factor VIII infusions are being counted 21 days post dosing.
Days post dosing = October 17, 2019 - dosing day.

SB-525 (PF-07055480) program transitioned to Pfizer for Phase 3 development

- Pfizer advancing SB-525 to Phase 3 in 2020
- Enrollment in Pfizer's Phase 3 lead-in study commenced in October
  - Objective: To establish ≥ 6 months of prospective efficacy data of current FVIII prophylaxis replacement therapy in the usual care setting of hemophilia A subjects, who are negative for nAb to SB-525 capsid (AAV6), prior to the Phase 3 gene therapy study

Sangamo THERAPEUTICS

+

Pfizer

Key takeaways

- Genomic medicine company building value with gene therapy, ex vivo gene-edited cell therapy, in vivo genome editing and genome regulation
- Precise, efficient and specific genomic medicine technology (ZFPs) backed by a robust patent estate
- Broad portfolio of rare and large indications across inherited metabolic diseases, immunology, CNS, hematology and oncology
- In-house cGMP facility and dedicated CDMO capacity provide manufacturing scale for clinical and commercial supply
- Strong balance sheet, five validating biopharma partnerships (Biogen, Kite, Pfizer, Sanofi, Takeda)

Catch The Next Wave

- Biotech is almost 40 years old (Genentech IPO 1983)
- Biotech is delivering on its promise over the next 25 years; demographics demand it; technology/innovation create it
- COVID-19 cements the critical need for biotech to battle future infectious threats

MTSL

Over 60 Years Of Combined Experience Uniquely Positions Us to Provide Quality, Unbiased Investment Advice

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Why You Need To Subscribe To MTSL

- Portfolio Performance
- BSA
- Breaking News
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